26 February 2015
EMA/CHMP/130684/2015
Press Office

Press release

Jinarc recommended for approval in rare kidney disease

Medicine to slow down cyst formation

The European Medicines Agency (EMA) has recommended granting a marketing authorisation to Jinarc (tolvaptan). Jinarc is indicated to slow the progression of cyst development and failing kidney function in adult patients with autosomal dominant polycystic kidney disease (ADPKD). Jinarc is for use in patients with normal to moderately reduced kidney function who have rapidly progressing ADPKD.

ADPKD affects approximately 4 in 10,000 people in the European Union (EU). It is an inherited condition marked by the growth of numerous fluid-filled cysts in the kidneys and other organs. The growth of cysts eventually affects kidney function and can cause the kidneys to fail. Symptoms include abdominal pain, problems with urinating, high blood pressure and infection.

No medicine is specifically authorised in the EU to treat patients with ADPKD. Current treatment focuses on the treatment of symptoms and complications. There is therefore a clear unmet need for an effective therapy for ADPKD.

Tolvaptan, a vasopressin-2-receptor antagonist, is already authorised in the EU for treating hyponatraemia (abnormally low sodium levels) although the doses studied in ADPKD are different.

Tolvaptan acts by blocking receptors in the kidneys to which the hormone vasopressin attaches, which regulates the level of water and sodium in the body. In ADPKD, it is thought that kidney cells do not respond normally to vasopressin, leading to the formation of fluid-filled cysts. By blocking vasopressin receptors in the kidneys, Jinarc can slow down cyst formation.

The positive opinion granted to Jinarc by the Committee for Medicinal Products for Human Use (CHMP) is based on a clinical trial in 1,445 adults with ADPKD which showed slower disease progression with Jinarc (as measured by enlargement of the kidneys and change in level of kidney function) compared with placebo over three years.

The CHMP recommended additional monitoring of the risk of liver damage with Jinarc, as this study found a greater number of people with serious liver adverse effects when taking Jinarc (2.3%, 22/961) compared with placebo (1.0%, 5/483). Although no cases of liver failure were found in this study, it is possible that in a wider population of patients with ADPKD tolvaptan may cause liver injury that could progress to liver failure.

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 8427 **Facsimile** +44 (0)20 3660 5555
E-mail press@ema.europa.eu **Website** www.ema.europa.eu

Jinarc is therefore proposed to be prescribed in the context of a registry to allow for additional monitoring, including blood tests to check the patient's liver function before starting treatment with Jinarc, and then repeated every month for 18 months and every three months thereafter. Additional safety profiling to evaluate further the risk of liver injury with the use of Jinarc will be carried out in a post-authorisation safety study. Jinarc must be initiated and monitored under the supervision of physicians with expertise in managing ADPKD and a full understanding of the risks of tolvaptan therapy including liver damage, and monitoring requirements.

Jinarc was designated as an orphan medicine and EMA provided protocol assistance to the applicant during the development of the medicine. Orphan designation and the associated incentives such as free scientific advice and protocol assistance are among the Agency's most important instruments to encourage the development of medicines for patients suffering from rare diseases.

The opinion adopted by the CHMP at its February 2015 meeting is an intermediary step on Jinarc's path to patient access. The CHMP opinion will now be sent to the European Commission for the adoption of a decision on EU-wide marketing authorisation. Once a marketing authorisation has been granted, a decision about price and reimbursement will then take place at the level of each Member State considering the potential role/use of this medicine in the context of the national health system of that country.

Notes

1. This press release, together with all related documents, is available on the Agency's website.
2. The applicant for Jinarc is Otsuka Pharmaceutical Europe Ltd.
3. Tolvaptan was approved in the EU in 2009 under the trade name Samsca. It is indicated for the treatment of adult patients with hyponatraemia secondary to syndrome of inappropriate antidiuretic hormone secretion (SIADH). The marketing authorisation holder is Otsuka Pharmaceutical Europe Ltd.
4. More information on the work of the European Medicines Agency can be found on its website: www.ema.europa.eu

Contact our press officer

Monika Benstetter

Tel. +44 (0)20 3660 8427

E-mail: press@ema.europa.eu